

TRANSKARYOTIC PRODUCTION AND DELIVERY OF DNASE

Abstract of the Disclosure

5 The invention relates to novel human DNA sequences,  
targeting constructs, and methods for producing novel genes  
encoding thrombopoietin, DNase I, and  $\beta$ -interferon by  
homologous recombination. The targeting constructs  
comprise at least: a) a targeting sequence; b) a regulatory  
sequence; c) an exon; and d) a splice-donor site. The  
10 targeting constructs, which can undergo homologous  
recombination with endogenous cellular sequences to  
generate a novel gene, are introduced into cells to produce  
homologously recombinant cells. The homologously  
recombinant cells are then maintained under conditions  
15 which will permit transcription of the novel gene and  
translation of the mRNA produced, resulting in production  
of either thrombopoietin, DNase I, or  $\beta$ -interferon. The  
invention further relates to a methods of producing  
pharmaceutically useful preparations containing  
20 thrombopoietin, DNase I, or  $\beta$ -interferon from homologously  
recombinant cells and methods of gene therapy comprising  
administering homologously recombinant cells producing  
thrombopoietin, DNase I, or  $\beta$ -interferon to a patient for  
therapeutic purposes.

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